

**BETH C. DRAIN, CA CSR NO. 7152**

BEFORE THE  
INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE  
AND THE  
APPLICATION REVIEW SUBCOMMITTEE  
TO THE  
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE  
ORGANIZED PURSUANT TO THE  
CALIFORNIA STEM CELL RESEARCH AND CURES ACT  
REGULAR MEETING

LOCATION: AS INDICATED ON THE AGENDA

DATE: JUNE 20, 2019  
11 A.M.

REPORTER: BETH C. DRAIN, CSR  
CA CSR. NO. 7152

FILE NO.: 2019-13

**BETH C. DRAIN, CA CSR NO. 7152**

**I N D E X**

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION:	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS (CLIN 1, 2 OR 3).	4
CLOSED SESSION	NONE
4. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO APPLICATIONS SUBMITTED IN RESPONSE TO AGENDA ITEM "3" ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
OPEN SESSION	
5. UPDATE ON PROCESSING OF APPLICATIONS.	22
6. PUBLIC COMMENT.	NONE
7. ADJOURNMENT.	31

**BETH C. DRAIN, CA CSR NO. 7152**

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JUNE 20, 2019; 11:00 A.M.

CHAIRMAN THOMAS: WELCOME, EVERYBODY, TO THE REGULAR MEETING OF THE ICOC AND APPLICATION REVIEW SUBCOMMITTEE FOR JUNE 2019. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES, HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: PRESENT.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: PRESENT.

MS. BONNEVILLE: LAURIE MILLER.

MS. MILLER: HERE.

MS. BONNEVILLE: ADRIANA PADILLA. JOE PANETTA. FRANCISCO PRIETO. ROBERT QUINT. AL ROWLETT.

MR. ROWLETT: PRESENT. IF YOU COULD ASK J.T., IT WAS A LITTLE DIFFICULT TO HEAR HIM ON THE PHONE.

MS. BONNEVILLE: GREAT. THANK YOU FOR THAT FEEDBACK.

**BETH C. DRAIN, CA CSR NO. 7152**

1 MS. BONNEVILLE: JEFF SHEEHY.

2 MR. SHEEHY: HERE.

3 MS. BONNEVILLE: OS STEWARD.

4 DR. STEWARD: HERE.

5 MS. BONNEVILLE: JONATHAN THOMAS.

6 CHAIRMAN THOMAS: HERE.

7 MS. BONNEVILLE: ART TORRES.

8 MR. TORRES: PRESENTO.

9 MS. BONNEVILLE: WHY, THANK YOU.

10 DIANE WINOKUR.

11 WE HAVE IS QUORUM.

12 CHAIRMAN THOMAS: OKAY. THANK YOU, MARIA.

13 WE HAVE ONE ITEM FOR ACTION TODAY. IT'S  
14 CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE  
15 TO CLINICAL TRIAL STAGE PROJECTS, CLINS 1, 2, OR 3.  
16 I'LL TURN THE MEETING AT THIS POINT OVER TO MR.  
17 SHEEHY.

18 MR. SHEEHY: THANK YOU, J.T. SO WHO'S  
19 GOING TO TAKE US THROUGH THIS TODAY? IS IT GOING TO  
20 BE DR. PATEL OR DR. SAMBRANO?

21 DR. PATEL: MR. SHEEHY, IT'S GOING TO BE  
22 ME, SHYAM.

23 MR. SHEEHY: OH, GREAT, SHYAM. WOULD YOU  
24 LIKE TO GO AHEAD WITH THE PRESENTATION?

25 DR. PATEL: THANK YOU, MR. SHEEHY.

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1 IT'S MY PLEASURE TO PRESENT THIS  
2 APPLICATION TO THE BOARD. I'M GOING TO DO A GENERAL  
3 INTRODUCTION FIRST AND THEN GET UNTIL THE DETAILS OF  
4 THE APPLICATION.

5 SO, AS YOU KNOW, THE CLINICAL STAGE  
6 FUNDING OPPORTUNITY IS COMPOSED OF THREE DISTINCT  
7 FUNDING OPPORTUNITIES. THE APPLICATION THAT IS IN  
8 FRONT OF YOU TODAY IS A CLIN2 CLINICAL TRIAL STAGE  
9 PROJECT.

10 WHEN THIS APPLICATION IS REVIEWED BY THE  
11 GRANTS WORKING GROUP, THEY WILL INDICATE THEIR  
12 PREFERENCE WITH THREE SCORES. IF IT'S GOT A TIER I  
13 SCORE, IT INDICATES IT HAS EXCEPTIONAL MERIT AND  
14 WARRANTS FUNDING. IF A GWG MEMBER THINKS THE  
15 APPLICATION DOES NOT WARRANT FUNDING AT THIS TIME,  
16 THEY CAN GIVE IT A SCORE OF 2 OR 3. A SCORE OF 2  
17 WOULD INDICATE THAT IT HAS SOME MINOR IMPROVEMENTS  
18 THAT CAN BE MADE IN A RESUBMISSION PROCESS. IF THEY  
19 THINK IT HAS SUFFICIENT FLAWS, IT CANNOT BE  
20 RESUBMITTED FOR SIX MONTHS, THEY WILL GIVE IT A  
21 SCORE OF 3.

22 AS YOU KNOW, WE STARTED THIS YEAR WITH A  
23 \$93 MILLION ALLOCATION FOR THE CLINICAL PROGRAM.  
24 THIS IS FOR THE GENERAL CLIN PROGRAM, BUT NOT  
25 INCLUDE SICKLE CELL PROJECT. AND OF THAT, 48

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1 MILLION HAVE BEEN APPROVED BY THE BOARD TO DATE.  
2 TODAY THE ONE APPLICATION UP FOR REVIEW IS  
3 REQUESTING \$12 MILLION. IF THAT IS FUNDED BY THE  
4 BOARD, THAT WOULD LEAVE \$33 MILLION GOING TOWARDS  
5 THE REMAINDER OF THE YEAR FOR THE CLIN PROGRAM.

6 CIRM HAS SET INTERNAL TARGETS FOR THE  
7 NUMBER OF CLIN2 AND CLIN1 PROJECTS THAT IT COULD  
8 HOPE TO GET APPROVED THIS YEAR. FOR CLIN2 THERE ARE  
9 FIVE APPROVED TO DATE, AND THIS WOULD MAKE THE SIXTH  
10 ONE OUT OF THE EIGHT THAT WERE TARGETED. AND THERE  
11 WERE TWO TARGETED FOR CLIN1, AND THOSE HAVE ALREADY  
12 BEEN APPROVED. AGAIN, THESE ARE NOT MEANT TO BE  
13 PROSCRIPTIVE, BUT INFORMATIVE.

14 SO THE APPLICATION TODAY, I'LL DESCRIBE  
15 THIS IN A LITTLE BIT OF DETAIL, IS CLIN2-11478, AND  
16 THE THERAPY ITSELF IS AUTOLOGOUS CTNS GENE-MODIFIED  
17 HEMATOPOIETIC STEM CELLS, AND THE INDICATION IS  
18 CYSTINOSIS. AND THE GOAL FOR THIS PROJECT IS TO  
19 COMPLETE A PHASE 1-2 CLINICAL TRIAL. AND THEY'RE  
20 REQUESTING ROUGHLY \$12 MILLION, AND THE MAXIMUM  
21 FUNDS ALLOWABLE FOR THIS CATEGORY IS ALSO \$12  
22 MILLION.

23 ON THIS NEXT SLIDE I HAVE SOME BASIC  
24 INFORMATION PREPARED FOR YOU TO HELP YOU ASSESS THE  
25 FUNDING OPPORTUNITY FOR THIS PARTICULAR APPLICATION.

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1 SO CYSTINOSIS IS A RELATIVELY RARE DISEASE THAT  
2 AFFECTS ONE IN A HUNDRED TO 200,000 NEWBORNS A YEAR.  
3 THAT'S WORLDWIDE. THE DISEASE ITSELF IS A LYSOSOMAL  
4 STORAGE DISEASE AND IT'S INHERITED. AND IT'S CAUSED  
5 BY A MUTATION OF THE CTNS GENE. THIS MUTATION  
6 RESULTS IN NO CYSTINOSIN PROTEIN PRESENT IN CELLS.  
7 THE CYSTINOSIN IS A TRANSMEMBRANE LYSOSOMAL PROTEIN  
8 THAT IS RESPONSIBLE FOR SHADOWING CYSTINE OUT OF  
9 LYSOSOME INTO THE CELLS. WITHOUT THAT, WHAT HAPPENS  
10 IS CYSTINE BUILDS UP IN THE LYSOSOME, RESULTING IN  
11 CRYSTAL FORMATION THAT CAN CAUSE DAMAGE IN VARIOUS  
12 CELLS WITHIN VARIOUS TISSUES AND ORGANS.

13 IN PARTICULAR, THE KIDNEY AND EYES ARE THE  
14 MOST VULNERABLE TO DAMAGE. IN THE MOST SEVERE OF  
15 CYSTINOSIS, WHICH THIS APPLICATION PROPOSES TO  
16 ADDRESS, IS EARLY ONSET AND RESULTS IN RENAL FANCONI  
17 SYNDROME, WHICH IS REDUCED FUNCTION OF THE KIDNEY AS  
18 WELL AS EVENTUAL KIDNEY FAILURE.

19 THERE IS NO CURE FOR CYSTINOSIS, BUT THERE  
20 IS A TREATMENT CURRENTLY AVAILABLE. THIS IS DAILY  
21 ORAL AND EYE ADMINISTRATION OF CYSTEAMINE. THIS  
22 DOES NOT ACTUALLY PREVENT RENAL FANCONI SYNDROME OR  
23 END-STAGE RENAL FAILURE.

24 THE PROPOSED GENE THERAPY HAS POTENTIAL TO  
25 BE A ONE-TIME TREATMENT OPTION. AND IN THE ANIMAL

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1 MODEL STUDY TO DATE IT HAS SHOWN TO MAINTAIN KIDNEY  
2 AND EYE FUNCTION. SO THERE IS POTENTIAL FOR IT TO  
3 DO THE SAME IN HUMANS.

4 THE ADDITIONAL THING I'D WANT TO NOTE IS  
5 THAT THE PROPOSED MECHANISM OF ACTION FOR THIS IS A  
6 LITTLE BIT DIFFERENT THAN PREVIOUS SIMILAR  
7 APPROACHES FOR LYSOSOMAL STORAGE DISORDERS. SO IN  
8 GENERAL FOR THOSE TYPES OF DISORDERS WHERE HSC'S  
9 HAVE BEEN USED, THE HSC PROGENY ARE TRANSFERRING  
10 SOLUBLE LYSOSOMAL PROTEINS TO THESE CELLS. IN THIS  
11 PARTICULAR INSTANCE, THE PROTEIN ITSELF IS A  
12 TRANSMEMBRANE LYSOSOMAL PROTEIN, AND IT IS BELIEVED  
13 THAT THE MECHANISM OF ACTION HERE IS CELL-CELL  
14 MEDIATED CONTACT FROM THE PROGENY OF THE HSC'S,  
15 WHICH ARE GOING TO BE MACROPHAGES. THE CELL-CELL  
16 CONTACT WITH THE CELLS IN THE VARIOUS TISSUES AND  
17 ORGANS AND ARE TRANSFERRING LYSOSOMAL PROTEIN TO  
18 THOSE CELLS. AND, THUS, IT COULD HAVE POTENTIAL  
19 APPLICATIONS FOR OTHER SIMILAR DISEASES AS WELL.

20 THIS IS A STEM CELL PROJECT BECAUSE, OF  
21 COURSE, IT INVOLVES GENE-MODIFIED HSC'S AND, THUS,  
22 THAT IS WHY IT IS ELIGIBLE FOR CIRM FUNDING.

23 WITH RESPECT TO OUR PORTFOLIO, THERE IS  
24 ONE IND STAGE ACTIVITY PROJECT FOR THE SAME DISEASE  
25 INDICATION, WHICH IS ACTUALLY THE EARLIER STAGE FOR

**BETH C. DRAIN, CA CSR NO. 7152**

1 THIS PARTICULAR PROJECT I'LL DESCRIBE IN THE NEXT  
2 SLIDE. WE DON'T HAVE ANY CLINICAL TRIAL STAGE FOR  
3 CYSTINOSIS IN OUR PORTFOLIO.

4 AS I MENTIONED, WE HAVE BEEN SUPPORTING  
5 THIS PROJECT SINCE IND-ENABLING STAGE WITH A CLIN1  
6 AWARD. THE CLIN1 AWARD AMOUNT WAS \$5.3 MILLION, ALL  
7 OF WHICH HAVE BEEN ISSUED TO DATE. THERE WERE FIVE  
8 MILESTONES SET FOR THIS PARTICULAR AWARD. THE  
9 APPLICANT HAS ACHIEVED THE GMP MANUFACTURING AND  
10 TECH TRANSFER MILESTONES ON TIME. THEY HAVE ALSO  
11 ACHIEVED PHARM-TOX SAFETY STUDIES THAT WERE  
12 IND-ENABLING AS WELL AS FILING OF THE IND AND TRIAL  
13 START-UP ACTIVITIES WITH MINOR DELAYS. THERE ARE  
14 ONGOING PHARM-TOX STUDIES, WHICH IS WHY THIS PROJECT  
15 IS STILL ACTIVE, THAT WILL BE COMPLETED AND  
16 SUBMITTED TO THE FDA AT THE APPROPRIATE TIME, AND  
17 THE FDA HAS AGREED TO THIS.

18 WHEN THE GWG REVIEWED THIS APPLICATION,  
19 ALL 15 SCORING MEMBERS GAVE IT A SCORE OF 1, MAKING  
20 IT A UNANIMOUS TIER I RECOMMENDATION FROM THE GWG.  
21 AND THE CIRM TEAM RECOMMENDATION CONCURS WITH THE  
22 GWG RECOMMENDATION TO FUND THIS APPLICATION FOR THE  
23 AWARD AMOUNT OF ROUGHLY \$12 MILLION. AND WITH THAT,  
24 I'M GOING TO HAND IT BACK TO MR. SHEEHY.

25 AND, MR. SHEEHY, I DO WANT TO NOTE THAT

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1 THE APPLICANT IS PRESENT HERE AT CIRM HEADQUARTERS  
2 AND IS ABLE TO SPEAK IF NEEDED.

3 MR. SHEEHY: THANK YOU, SHYAM.

4 SO DO I HAVE A MOTION TO EITHER ACCEPT OR  
5 REJECT THE TEAM RECOMMENDATION AND TO FUND OR NOT  
6 FUND IT?

7 DR. HIGGINS: I MOVE TO ACCEPT.

8 MR. SHEEHY: OKAY. TO FUND. AND DO I  
9 HAVE A SECOND?

10 CHAIRMAN THOMAS: SECOND.

11 MR. SHEEHY: THE MOTION IS BY DR. HIGGINS  
12 AND WHO IS THE SECOND BY?

13 CHAIRMAN THOMAS: J.T.

14 MR. SHEEHY: OKAY.

15 CHAIRMAN THOMAS: DR. QUINT, YES.

16 MR. SHEEHY: DR. QUINT. I'M SORRY.  
17 GREAT.

18 IS THERE ANY BOARD DISCUSSION ABOUT THIS  
19 APPLICATION?

20 DR. MARTIN: THIS IS DAVE. I HAVE A  
21 QUESTION. THE SUBJECTS, THE PATIENTS, WHO WILL BE  
22 CANDIDATES FOR INCLUSION, THOSE ARE ADULT CYSTINOTIC  
23 PATIENTS OR PEDIATRIC OR NEWBORNS? WHAT POPULATION?

24 DR. PATEL: THE EVENTUAL TARGET POPULATION  
25 FOR THIS WOULD BE PEDIATRIC PATIENTS. IN THE TRIAL,

**BETH C. DRAIN, CA CSR NO. 7152**

1 WE ARE LOOKING THAT INFORMATION UP AND WILL PROVIDE  
2 IT TO YOU IN A MINUTE. WE ALSO HAVE THE APPLICANT  
3 HERE WHO CAN RESPOND TO THAT QUESTION IF YOU WOULD  
4 LIKE.

5 MR. SHEEHY: WE USUALLY WAIT TILL PUBLIC  
6 COMMENT, BUT I THINK J.T. IS RUNNING THE MEETING  
7 NOW. SO WE WILL GO AHEAD AND LET THE APPLICANT  
8 SPEAK.

9 MS. BONNEVILLE: JEFF, I THINK A FEW BOARD  
10 MEMBERS HAVE JOINED THE CALL. SO I'M JUST GOING TO  
11 CALL THEIR NAMES IF THAT'S OKAY WITH YOU.

12 DR. DEAS, ARE YOU ON THE LINE?

13 DR. DEAS: YES. HERE.

14 MS. BONNEVILLE: THANK YOU. DR. MELMED.  
15 AND DR. QUINT IS OBVIOUSLY ON THE LINE. SO THANK  
16 YOU. OKAY.

17 DR. PATEL: SO TO RESPOND TO THE QUESTION,  
18 IN THIS PARTICULAR TRIAL, THE PATIENT WOULD HAVE TO  
19 BE OVER 18 YEARS OF AGE.

20 MR. SHEEHY: DR. MARTIN, DOES THAT ANSWER  
21 YOUR QUESTION? OR DO YOU HAVE QUESTIONS FOR THE  
22 APPLICANT? SHOULD WE BRING THE APPLICANT UP FOR ANY  
23 QUESTIONS?

24 DR. MARTIN: I COULDN'T UNDERSTAND THAT.

25 DR. PATEL: ADULT PATIENTS.

**BETH C. DRAIN, CA CSR NO. 7152**

1 DR. MARTIN: ADULT. OKAY. THAT'S OVER  
2 18? AND WHAT'S THE MAXIMUM AGE?

3 DR. PATEL: THERE IS NO MAXIMUM AGE IN THE  
4 INCLUSION CRITERIA.

5 DR. MARTIN: THANK YOU.

6 DR. DULIEGE: THIS IS ANNE-MARIE. AND I A  
7 HAVE A CLARIFICATION QUESTION. THE CIRM BOARD HAS  
8 FUNDED APPROXIMATELY \$5.3 MILLION FOR THIS VERY  
9 PROJECT, WHICH LED TO TRIALS PRODUCT. IS IT THE  
10 VERY SAME TRIAL FOR WHICH WE ARE CONSIDERING FUNDING  
11 \$12 MILLION OR IS THAT ANOTHER TRIAL?

12 DR. PATEL: FOR THE CLIN1 AWARD, WE DO  
13 ALLOW THE APPLICANT TO DO SOME TRIAL START-UP  
14 ACTIVITIES, BUT DOES NOT ACTUALLY INVOLVE TREATING  
15 THE PATIENTS. SO NO PATIENTS HAVE BEEN TREATED WITH  
16 CIRM FUNDING TO DATE FOR THIS TRIAL.

17 DR. DULIEGE: GREAT. THANK YOU.

18 DR. PATEL: IT'S THE PRECURSOR TO THIS  
19 AWARD.

20 DR. DULIEGE: I UNDERSTAND. AND MAYBE CAN  
21 WE GET A LITTLE BIT MORE DETAIL ABOUT THE EYE LEVEL,  
22 THE STUDY DESIGN, THE NUMBER OF PATIENTS, THEIR  
23 CONDITION TO BEGIN WITH, AND THE INTERVENTION AND  
24 THE FOLLOW-UP?

25 DR. PATEL: IF IT'S OKAY WITH THE BOARD,

**BETH C. DRAIN, CA CSR NO. 7152**

1 I'D LIKE TO HAVE THE APPLICANT RESPOND TO THAT  
2 QUESTION.

3 DR. DULIEGE: THAT'S GREAT.

4 DR. CHERQUI: THANK YOU. SO I'M STEPHANIE  
5 CHERQUI, AND I AM THE PRINCIPAL INVESTIGATOR ON THIS  
6 GRANT. SO I WANT TO CLARIFY A COUPLE OF PATIENTS.  
7 WE HAD A SECOND DESIGN ELEMENT. SO THE FIRST COHORT  
8 WILL BE TWO ADULTS, AND THE SECOND COHORT IS TWO  
9 ADULTS, AND FOR THE THIRD COHORT OF TWO PATIENTS, IF  
10 THE RISK ASSESSMENT IS GOOD, WE ENROLL AS OF THIS  
11 TIME. THAT'S JUST CLARIFICATION.

12 SO WE HAVE THE PATIENT COMING FOR  
13 TRAINING, AND THEN WE HAD A BASELINE STUDY WHICH  
14 WILL EVALUATE ALL THE TISSUES BECAUSE THIS DISEASE  
15 AFFECTS ALL THE TISSUES OF THE BODY, THE KIDNEY, THE  
16 EYE, THE LEFT SIDE OF THE BRAIN, (UNINTELLIGIBLE).  
17 SO WE NEEDED TO GO THROUGH A MAJOR STUDY OF ALL THE  
18 COMPLICATIONS. AND SO WE HAVE A BASELINE  
19 (UNINTELLIGIBLE). THEN WE WILL COLLECT  
20 (UNINTELLIGIBLE) AND WILL BE MANUFACTURED BY UCLA BY  
21 DONALD KOHN. AND THIS WILL TAKE TWO, THREE MONTHS.  
22 AND AFTER WE'LL COME BACK, THE PATIENTS WILL COME  
23 BACK FOR TRANSPLANTATION. AND THEN AFTER  
24 TRANSPLANTATION, THEY WILL HAVE A FOLLOW-UP VISIT TO  
25 ASSESS SAFETY, BUT ALSO EFFICACY BECAUSE IT'S A

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1 PHASE 1-2 CLINICAL TRIAL. WE WILL HAVE FOLLOW-UP  
2 VISITS EVERY SIX MONTHS FOR THREE YEARS.

3 DR. DULIEGE: THANK YOU.

4 DR. MARTIN: THIS IS DAVE. I HAVE SOME  
5 OTHER QUESTIONS SINCE THE APPLICANT IS PRESENT. AND  
6 THAT IS THESE ADULT PATIENTS PRESUMABLY ARE GOING TO  
7 HAVE SOME DEGREE OF FANCONI'S. AND WHAT EVIDENCE DO  
8 YOU HAVE OF THE REVERSIBILITY IN EXPERIMENTAL  
9 ANIMALS OF THE RENAL DISEASE? AND IS THAT GOING TO  
10 BE AN ENDPOINT IN THE STUDY AND THE INITIAL STUDY?

11 DR. CHERQUI: THESE ARE VERY GOOD POINTS.  
12 SO WE HAVE EVENTS THAT IF PATIENT HAS FANCONI  
13 SYNDROME, WE DO A REVERSE BY THIS TREATMENT BECAUSE  
14 THE FANCONI SYNDROME IS NOT JUST THE ACTUATION OF  
15 CYSTINE, BUT DUE THE ABSENCE PROTEIN. AND WE SHOWED  
16 BY OUR MECHANISM OF ACTION THAT WE CAN RESTORE THE  
17 PROTEIN INTO THE KIDNEY CELLS. SO WE SHOWED THAT IN  
18 MICE WE CAN REVERSE THIS. THE SECOND IMPORTANT AND  
19 (UNINTELLIGIBLE) OF THE EYE, WE SHOW THAT WE CAN  
20 CLEAR THE CYSTINE CRYSTALS IN THE CORNEA WHICH  
21 RESULTS IN BETTER EYE FUNCTION.

22 AND I WANT TO POINT OUT THAT THERE WAS AN  
23 ALLOGENEIC TRANSPLANT THAT WAS PERFORMED IN BELGIUM.  
24 IT JUST GOT BACK AND IT WAS RECENTLY PUBLISHED.  
25 UNFORTUNATELY, THIS KID WAS 16 YEARS OLD AND HE DID

**BETH C. DRAIN, CA CSR NO. 7152**

1 NOT PROGRESS BECAUSE IT WAS AN ALLOGENEIC  
2 TRANSPLANT. AND HE STAYED IN THE HOSPITAL FOR MORE  
3 THAN A YEAR AND EVENTUALLY DIED BECAUSE OF THE  
4 (UNINTELLIGIBLE) THAT DEVELOPED. AND IN SPITE OF  
5 THIS SAD AND UNFORTUNATE CASE, THE PHYSICIAN SHOULD  
6 SHOW THAT THERE WAS IMPROVEMENT OF THE FANCONI  
7 SYNDROME, STABILIZATION OF THE KIDNEY FUNCTION, AND  
8 THE EYE DEFECT. SO THE PHOTOPHOBIA, BECAUSE OF THE  
9 CORNEA CRYSTAL, WAS COMPLETELY RESOLVED. THE  
10 PATIENT HAD A GREAT PHOTOPHOBIA TO NO PHOTOPHOBIA  
11 TALL.

12 SO BECAUSE OF ALL THIS EVIDENCE OF OUR  
13 STUDIES AND BECAUSE OF THIS CASE, SECOND CASE, WE  
14 BELIEVE THAT THE KIDNEY AND THE EYE WILL BE VERY  
15 STRONG ENDPOINT. IF WE HAVE OTHER ENDPOINT, WE WILL  
16 STUDY THE FUNCTION, HALF FUNCTION, AND WE REALLY  
17 GO -- WE STUDY ALL THE COMPLICATIONS.

18 DR. MARTIN: HOW MANY SUBJECTS -- THANK  
19 YOU. AND HOW MANY SUBJECTS, PATIENTS, WOULD FIT  
20 YOUR INCLUSION OR SATISFY YOUR INCLUSION CRITERIA IN  
21 THE STATE?

22 DR. CHERQUI: SO WE ARE TRYING WITH SIX  
23 PATIENTS, AND SO FAR WE HAVE MANY PATIENT  
24 VOLUNTEERS, AND I'M HAPPY TO REPORT THAT WE HAVE OUR  
25 FIRST PATIENT THAT WILL START IN JULY 8. SO IT'S

**BETH C. DRAIN, CA CSR NO. 7152**

1 COMING, AND ACTUALLY IT STARTS JULY 8TH, AND IT'S A  
2 20 YEARS OLD BOY WITH NO KIDNEY TRANSPLANT AND  
3 PRETTY HEALTHY. SO IT'S A REALLY GOOD FIRST  
4 PATIENT. WE HAVE MANY VOLUNTEERS ALREADY, AND SO  
5 IT'S A MATTER OF TREATING THEM, WHICH I'M TRYING NOT  
6 TO BE -- WE HAVE TWO CLINICAL PI THAT HAVE DONE OUR  
7 TRANSPLANTATION AND A MEDICAL DOCTOR IN CYSTINOSIS.  
8 SO WE HAVE A CHOICE THAT WE HAVE MANY CANDIDATES.

9 DR. MARTIN: THANK YOU.

10 MR. SHEEHY: ARE THERE OTHER BOARD  
11 COMMENTS OR QUESTIONS FOR THE APPLICANT? ARE THERE  
12 ANY --

13 MS. BONNEVILLE: THERE AREN'T ANY  
14 QUESTIONS HERE.

15 MR. SHEEHY: IS THERE ANY PUBLIC COMMENT  
16 AT ANY OF THE SITES?

17 DR. CHERQUI: I HAVE PREPARED SOMETHING,  
18 HAVE A POINT I WANTED TO SAY. SO I JUST WANT TO ADD  
19 SOMETHING. I WOULD REALLY SINCERELY THANK THE CIRM  
20 BECAUSE OF THE T-1 GRANT THAT WE RECEIVED IN 2016.  
21 BECAUSE OF THIS GRANT, WE WERE ABLE TO DO ALL THE  
22 STUDY AND PREPARE THE IND IN A RELATIVELY SHORT TIME  
23 OF THREE YEARS, WHICH WOULD HAVE BEEN COMPLETELY  
24 IMPOSSIBLE WITHOUT CIRM FUNDING. SO I WANTED TO  
25 TAKE THIS OPPORTUNITY TO SAY THANK YOU FOR THAT.

**BETH C. DRAIN, CA CSR NO. 7152**

1 I ALSO WANTED TO POINT OUT THAT I HAVE AN  
2 OUTSTANDING TEAM THAT TAKE CARE OF THIS CLINICAL  
3 TRIAL. WE HAVE THE CRITICAL SUPPORT OF THE UCSD  
4 SIGHT CLINIC AND THE (UNINTELLIGIBLE) CENTER. WE  
5 HAVE AN AMAZING MEDICAL TEAM OF 12 PHYSICIAN WHO ARE  
6 VERY DEDICATED, AND I'M EXCITED (UNINTELLIGIBLE).  
7 AND WE HAVE, AS I SAID, MANUFACTURING OF THE PRODUCT  
8 THAT IS DONE BY DONALD KOHN, WHO IS WELL KNOWN BY  
9 CIRM, AND WE ARE (UNINTELLIGIBLE) ON THIS PROJECT.

10 AND I JUST WANT TO SHARE AND POINT OUT THE  
11 MECHANISM OF ACTION THAT WE SHOWED ALLOWED US TO  
12 APPLY THIS PROOF OF CONCEPT TO ALL THE OTHER  
13 DISORDER THAT WAS NOT SOUGHT AS AN OPTION FOR THIS  
14 KIND OF TREATMENT. AND WE HAVE ALREADY SHOWN --  
15 SUCCESSFULLY SHOWN THE PROOF OF CONCEPT ON ANOTHER  
16 (UNINTELLIGIBLE) DISORDER, BALINT DISEASE, AND WHICH  
17 WE RECEIVED RECENTLY A QUEST CIRM GRANT. AND THIS  
18 PATIENT GOT A HEART TRANSPLANT. AND SO WE SHOWED  
19 THAT STEM CELL INTERACT, AND WE SHALL TRY THAT ON  
20 FRIEDREICH ATAXIA, WHICH IS (UNINTELLIGIBLE)  
21 DISORDER FOR WHICH THERE IS NO TREATMENT.

22 SO I THINK THAT IF THIS TRIAL IS  
23 SUCCESSFUL, THERE IS MANY APPLICATION THAT WE COULD  
24 WORK. SO THANK YOU FOR YOUR TIME.

25 I THINK ALSO WANTED TO DO A QUICK OTHER

**BETH C. DRAIN, CA CSR NO. 7152**

1 COMMENT. NANCY STACK IS THE DIRECTOR OF THE AMAZING  
2 CYSTINOSIS RESEARCH FOUNDATION WHO HAS WORKED WITH  
3 US SINCE THE BEGINNING OF THIS PROJECT.

4 MS. STACK: HELLO. MY NAME IS NANCY  
5 STACK. AND I WANT TO THANK YOU FOR ALLOWING ME TO  
6 SPEND A MINUTE OR TWO TO TALK ABOUT CYSTINOSIS.

7 I'M ARE THE FOUNDER AND PRESIDENT OF THE  
8 CYSTINOSIS RESEARCH FOUNDATION. OUR DAUGHTER  
9 NATALIE WAS DIAGNOSED WITH CYSTINOSIS WHEN SHE WAS  
10 AN INFANT. CYSTINOSIS, AS YOU KNOW NOW, IS A RARE  
11 GENETIC DISEASE THAT'S CHARACTERIZED BY THE ABNORMAL  
12 ACCUMULATION OF CYSTINE IN EVERY CELL IN THE BODY.  
13 THE BUILDUP OF CYSTINE DESTROYS EVERY ORGAN IN THE  
14 BODY INCLUDING THE KIDNEYS, EYES, LIVER, THYROID,  
15 AND BRAIN. THE AVERAGE DEATH FROM CYSTINOSIS AND  
16 ITS COMPLICATIONS IS 28 YEARS OF AGE. FOR CHILDREN  
17 AND ADULTS WITH CYSTINOSIS, THERE ARE NO HEALTHY  
18 DAYS. THEY TAKE BETWEEN 8 TO 12 MEDICATIONS AROUND  
19 THE CLOCK EVERY DAY JUST TO STAY ALIVE. NATALIE  
20 TAKES 45 PILLS A DAY. IT'S A RELENTLESS AND  
21 DEVASTATING DISEASE.

22 MEDICAL COMPLICATIONS ABOUND, AND OUR  
23 CHILDREN'S LIVES ARE FILLED WITH A MYRIAD OF  
24 SYMPTOMS AND TREATMENTS. THERE ARE FEED TUBE  
25 FEEDINGS, KIDNEY TRANSPLANTS, BONE PAIN, DAILY

**BETH C. DRAIN, CA CSR NO. 7152**

1 VOMITING, SWALLOWING DIFFICULTIES, MUSCLE WASTING,  
2 SEVERE GASTROINTESTINAL SIDE EFFECTS, AND FOR SOME  
3 BLINDNESS.

4 WE STARTED THE FOUNDATION IN 2003. WE  
5 HAVE WORKED WITH AND FUNDED DR. STEPHANIE CHERQUI  
6 SINCE 2006. AS A FOUNDATION, OUR RESOURCES ARE  
7 LIMITED, BUT WE WERE ABLE TO FUND THE INITIAL GRANT  
8 FOR STEPHANIE'S STEM CELL STUDIES. WHEN CIRM  
9 AWARDED A GRANT TO STEPHANIE IN 2016, IT ALLOWED HER  
10 TO COMPLETE THE STUDIES, FILE THE IND, AND AS A  
11 RESULT WE NOW HAVE FDA APPROVAL FOR THE CLINICAL  
12 TRIALS. YOUR SUPPORT HAS CHANGED THE COURSE OF THIS  
13 DISEASE.

14 WHEN THE FDA APPROVED THE CLINICAL TRIAL  
15 FOR CYSTINOSIS LATE LAST YEAR, OUR COMMUNITY WAS  
16 FILLED WITH THE RENEWED SENSE OF HOPE AND OPTIMISM.  
17 I HEARD FROM 32 ADULTS WITH CYSTINOSIS, ALL OF THEM  
18 INTERESTED IN THE CLINICAL TRIALS. OUR ADULTS KNOW  
19 THAT THIS IS THEIR ONLY CHANCE TO LIVE A FULL LIFE.  
20 WITHOUT THIS TREATMENT, THEY WILL DIE FROM  
21 CYSTINOSIS. IN EVERY E-MAIL I RECEIVED, THERE WAS A  
22 MESSAGE OF HOPE AND GRATITUDE.

23 I RECEIVED AN E-MAIL FROM A YOUNG WOMAN  
24 WHO SAID THIS: "IT'S A NEW AWAKENING TO LEARN THIS  
25 MORNING THAT HUMAN CLINICAL TRIALS HAVE BEEN

**BETH C. DRAIN, CA CSR NO. 7152**

1 APPROVED BY THE FDA. I REITERATE MY IMMENSE  
2 INTEREST TO PARTICIPATE IN THIS TRIAL AS SOON AS  
3 POSSIBLE BECAUSE MY QUALITY OF LIFE IS AT A LOW EBB  
4 AND THE TRIAL IS REALLY MY ONLY HOPE. TIME IS  
5 RUNNING OUT."

6 AND A MOM OF A 19-YEARS-OLD YOUNG MAN WHO  
7 WANTS TO BE THE FIRST PATIENT IN THE TRIAL WROTE AND  
8 SAID THIS: "ON THE DAY THE TRIAL WAS ANNOUNCED, I  
9 STARTED TO CRY TEARS OF PURE HAPPINESS. AND I  
10 THOUGHT, ANOTHER SUMMER JUST TO WAKE UP AND HAVE A  
11 CHILD WHO WILL NO LONGER HAVE CYSTINOSIS. I FELT SO  
12 HAPPY FOR WHOMEVER THAT MOM WOULD BE. I NEVER  
13 IMAGINED THE MOM I WAS THINKING ABOUT COULD BE ME.  
14 I'M SO HUMBLLED TO HAVE THIS OPPORTUNITY FOR MY SON  
15 TO TRY TO LIVE DISEASE FREE."

16 MY OWN DAUGHTER NATALIE WENT INTO MY ARMS  
17 THAT DAY AND WE CRIED TEARS OF JOY. FINALLY, THE  
18 HOPE WE HAD CLUNG TO WAS NOW A REALITY. WE HAD COME  
19 FULL CIRCLE. I ASKED NATALIE HOW IT FELT TO KNOW  
20 THAT SHE COULD BE CURED. AND SHE SAID, "I SPENT MY  
21 ENTIRE LIFE THINKING THAT I WOULD DIE FROM  
22 CYSTINOSIS IN MY 30S, BUT NOW I MIGHT LIVE A FULL  
23 LIFE AND I'M THINKING ABOUT HOW MUCH THAT CHANGES  
24 HOW I THINK ABOUT MY FUTURE. I NEVER PLAN TOO FAR  
25 AHEAD, BUT NOW I CAN."

**BETH C. DRAIN, CA CSR NO. 7152**

1 AS A MOTHER, WORDS CAN'T POSSIBLY CONVEY  
2 WHAT IT FEELS LIKE TO KNOW THAT MY CHILD HAS A  
3 CHANCE TO LIVE A LONG, HEALTHY LIFE FREE OF  
4 CYSTINOSIS. I CAN BREATHE AGAIN. ON BEHALF OF ALL  
5 THE CHILDREN AND ADULTS WITH CYSTINOSIS, THANK YOU  
6 FOR FUNDING DR. CHERQUI, FOR CARING ABOUT OUR  
7 COMMUNITY, FOR VALUING OUR CHILDREN, AND FOR MAKING  
8 THIS TREATMENT A REALITY. OUR COMMUNITY IS READY TO  
9 START THIS TRIAL. THANK YOU FOR MAKING IT HAPPEN.  
10 THANK YOU.

11 MR. SHEEHY: THANK YOU. IS THERE  
12 ADDITIONAL PUBLIC COMMENT? SO COULD WE CALL THE  
13 ROLL ON THE MOTION TO ACCEPT THE TEAM RECOMMENDATION  
14 AND FUND THIS APPLICATION?

15 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

16 DR. DULIEGE: YES.

17 MS. BONNEVILLE: DAVID HIGGINS.

18 DR. HIGGINS: YES.

19 MS. BONNEVILLE: STEVE JUELSGAARD.

20 MR. JUELSGAARD: YES.

21 MS. BONNEVILLE: DAVE MARTIN.

22 DR. MARTIN: YES.

23 MS. BONNEVILLE: LAUREN MILLER.

24 MS. MILLER: YES.

25 MS. BONNEVILLE: ROBERT QUINT.

**BETH C. DRAIN, CA CSR NO. 7152**

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DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: MOTION CARRIES.

MR. SHEEHY: THAT CONCLUDES THE BUSINESS  
OF THE APPLICATION REVIEW SUBCOMMITTEE.

MS. BONNEVILLE: THANK YOU, JEFF.

CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.

WE HAVE ONE OTHER ITEM WHICH IS NOT AN  
ACTION ITEM TO DISCUSS WITH MEMBERS OF THE ARS. AS  
WE'VE BEEN DISCUSSING IN SOME DETAIL FOR QUITE A  
LENGTH OF TIME, THE TIME WAS GOING TO COME WHEN THE  
FUNDING AVAILABLE FOR NEW AWARDS WAS GOING TO RUN  
OUT AND THAT THAT WOULD PRECIPITATE NOT MAKING  
FURTHER NEW CLIN APPLICATIONS. WE TALKED ABOUT THIS  
AT OUR LAST BOARD MEETING, AND I THINK SHERRY, AMONG  
OTHERS, SAID THAT WE NEED TO MAKE SURE WE ARE

**BETH C. DRAIN, CA CSR NO. 7152**

1 NOTIFYING THE GRANTEE COMMUNITY WHAT THE STATE OF  
2 PLAY IS WITH AVAILABLE FUNDING AND WHEN WE WOULD  
3 HAVE TO STOP TAKING FURTHER APPLICATIONS PENDING  
4 RAISING FURTHER BRIDGE FUNDING.

5 AND SO THAT TOPIC IS NOW RIPE, HAVING  
6 TAKEN STOCK OF WHAT IS IN THE PIPELINE NOW TO BE  
7 REVIEWED IN THE NEXT ONE OR TWO GWG'S AND AS A  
8 RESULT OF THAT AND THE REAL POSSIBILITY THAT AWARDS  
9 THROUGH WHAT WILL BE REVIEWED IN AUGUST COULD  
10 POSSIBLY EXCEED WHAT WE HAVE AVAILABLE FOR FUNDING.  
11 WE HAVE AN UPDATE, WHICH IS ITEM NO. 5, ON THIS  
12 SUBJECT, AND THAT WILL BE DISCUSSED BY DR. SAMBRANO.

13 DR. SAMBRANO: THANK YOU, DR. THOMAS.

14 SO AS WAS MENTIONED, WE ARE TRYING TO KEEP  
15 A CLOSE EYE, PARTICULARLY THIS YEAR, ON OUR OVERALL  
16 BUDGET FOR THE CLINICAL PROGRAM. AND SO TRYING TO  
17 DETERMINE AND PREDICT, TO THE EXTENT THAT WE CAN,  
18 APPLICATIONS THAT ARE COMING IN, THOSE THAT ARE  
19 LIKELY TO GET APPROVED, AND WHEN THAT BUDGET IS  
20 LIKELY TO BE DEPLETED.

21 SO IN THE MEMO WE SUMMARIZE WHAT IS THE  
22 CURRENT STATE OF THE PROGRAM WITH MODIFICATIONS.  
23 BASED ON THE APPROVAL OF THE APPLICATION TODAY, WE  
24 HAVE 33 MILLION NOW THAT REMAIN IN THE CLINICAL  
25 BUDGET, AND WE HAVE SEVERAL APPLICATIONS THAT ARE IN

**BETH C. DRAIN, CA CSR NO. 7152**

1 THE SYSTEM, MEANING THAT THEY ARE IN THE PROCESS OF  
2 REVIEW IN SOME WAY. SO WE HAVE A COUPLE OF  
3 APPLICATIONS THAT WILL BE REVIEWED NEXT TUESDAY BY  
4 THE GWG. WE HAVE WHAT WE EXPECT TO BE EIGHT  
5 APPLICATIONS THAT WILL BE REVIEWED IN JULY, WHICH  
6 INCLUDE SOME TIER II'S THAT ARE COMING BACK. SO  
7 WITH ALL OF THAT, WE HAVE ABOUT 76 MILLION TOTAL  
8 REQUESTS FROM ALL OF THOSE APPLICATIONS.

9 IN ADDITION TO THOSE, WE EXPECT  
10 APPLICATIONS TO COME IN AT THE END OF THIS MONTH,  
11 PROBABLY UP TO FOUR, AND WE DON'T KNOW WHAT THOSE  
12 BUDGETS SPECIFICALLY WILL BE, BUT THEY WOULD ADD TO  
13 THE 76 MILLION AND COULD CARRY IT OVER TO ABOVE 90  
14 MILLION.

15 SO GIVEN THOSE FACTORS, WHAT WE HAVE  
16 DECIDED TO DO IS, ONE, IS ALERT APPLICANTS OF THE  
17 POSSIBILITY THAT EVEN IF THEY APPLY AT THE END OF  
18 THIS MONTH, THERE MAY BE NO FUNDS AVAILABLE BY THE  
19 TIME THEY REACH THE END OF THE REVIEW CYCLE. AND  
20 ALSO THAT, ASSUMING THIS CONTINUES TO HOLD, THAT WE  
21 WOULD NOT HAVE AN OPEN APPLICATION FOR THE END OF  
22 JULY, WHICH IS THE TYPICAL END-OF-THE-MONTH DEADLINE  
23 IN JULY, MAY NOT HAPPEN IF WE FEEL THAT WE ARE  
24 LIKELY TO DEplete FUNDS.

25 SO THAT'S A SUMMARY OF THE UPDATE AND

**BETH C. DRAIN, CA CSR NO. 7152**

1 HAPPY TO TAKE ANY QUESTIONS IF YOU HAVE THEM.

2 CHAIRMAN THOMAS: DO MEMBERS OF THE ARS  
3 HAVE QUESTIONS FOR DR. SAMBRANO?

4 MR. SHEEHY: I ACTUALLY HAVE A QUESTION.  
5 SO HOW ARE WE NOTIFYING APPLICANTS? ARE WE PUTTING  
6 THE WORD ON THE WEBSITE?

7 DR. SAMBRANO: SO WE ARE DOING IT -- SO  
8 THERE'S TWO PLACES. THE PRIMARY PLACE IS IN THE  
9 WEBSITE WHERE THE APPLICATION IS. SO ANYBODY WHO  
10 INTENDS TO SUBMIT OR OPEN AN APPLICATION WILL SEE IT  
11 IN THE FACE PAGE OF THAT AREA. WE ALSO INTEND TO  
12 POST IT IN THE PUBLIC WEBSITE PAGE JUST TO ALERT  
13 ANYBODY WHO IS COMING ANEW TO SEE THE PROGRAM  
14 ANNOUNCEMENTS. AND THEN FOR THOSE THAT WE HAPPEN TO  
15 KNOW THAT ARE EITHER IN CONTACT WITH CIRM SCIENCE  
16 OFFICERS, WE ARE HAVING THEM ALSO ALERT THEM OF WHAT  
17 THE SITUATION IS SO THAT THEY CAN DECIDE  
18 APPROPRIATELY WHAT TO DO.

19 DR. STEWARD: SO THIS IS OS. COULD I MAKE  
20 A COMMENT?

21 CHAIRMAN THOMAS: SURE.

22 DR. STEWARD: THIS REALLY FOLLOWS UP ON  
23 WHAT SHERRY HAD SAID AT OUR LAST DISCUSSION OF THIS,  
24 WHICH IS THAT WE NEED TO BE CONSCIOUS OF THE FACT  
25 THAT A LOT OF PEOPLE OUT THERE HAVE BEEN WORKING

**BETH C. DRAIN, CA CSR NO. 7152**

1 HARD TO PREPARE APPLICATIONS AND THAT JUST SHUTTING  
2 OFF THE SPIGOT COMPLETELY AND IN SUCH A VERY SHORT  
3 TIME FRAME COULD BE HIGHLY DISRUPTIVE. THAT'S MY  
4 CONCERN.

5 OBVIOUSLY IT DOES NOT MAKE SENSE EITHER TO  
6 CONTINUE TO ACCEPT APPLICATIONS WHEN WE KNOW THERE'S  
7 NOT GOING TO BE MONEY AVAILABLE BECAUSE THAT AT  
8 LEAST IMPLIES TO THE INVESTIGATORS THAT THERE IS  
9 SOME CHANCE OF FUNDING. SO I'VE BEEN THINKING ABOUT  
10 THIS, AND I HAVEN'T ACTUALLY BEEN ABLE TO COMPLETELY  
11 FORMULATE AN IDEA, BUT BY WAY OF INTRODUCTION, THOSE  
12 ARE MY CONCERNS.

13 AND JUST TO OFFER A VERY PRELIMINARY AND  
14 NOT VERY WELL THOUGHT OUT ALTERNATIVE OR MAYBE  
15 MODIFICATION TO WHAT'S BEING PROPOSED HERE, WHAT I  
16 WOULD SUGGEST IS SOMETHING MORE LIKE A PAUSE. SO  
17 LET ME UNPACK THAT JUST A BIT.

18 RATHER THAN SAYING WE ARE NOT GOING TO  
19 ACCEPT ANY MORE APPLICATIONS AFTER JULY, INDICATE  
20 THAT WE WILL NOT ACCEPT APPLICATIONS IN THE NORMAL  
21 TIME FRAME IN JULY. HOWEVER, WE WILL RECONSIDER THE  
22 SITUATION WHEN, FOR EXAMPLE, SEPTEMBER WHEN THE FULL  
23 BOARD GETS A CHANCE TO MEET. THAT DELAY OR PAUSE,  
24 IF YOU WANT, WILL GIVE US TIME TO ASSESS THE EXTENT  
25 TO WHICH ANY FUNDS COME BACK TO CIRM BECAUSE OF

**BETH C. DRAIN, CA CSR NO. 7152**

1 PROJECTS THAT HAVE FAILED TO MEET MILESTONES, WOULD  
2 HAVE A BETTER IDEA OF THE OVERALL BUDGET SITUATION  
3 FOR THE YEAR. THERE MAY BE OTHER CONDITIONS AND  
4 CONSIDERATIONS AS WELL; FOR EXAMPLE, WE WILL BE  
5 CONSIDERING THE DISTRIBUTION OF FUNDS INTO DIFFERENT  
6 BUCKETS. SO RIGHT NOW WE ARE LOOKING AT THE CLIN  
7 BUDGET AND THE TRAN BUDGET, AND IT MAY BE  
8 APPROPRIATE TO ACTUALLY RECONSIDER EXACTLY HOW MUCH  
9 IS THERE. THAT ALL COULD BE DONE IN SEPTEMBER WHEN  
10 WE HAVE A MUCH BETTER IDEA.

11 THE THIRD REASON FOR DOING THIS IS THAT IF  
12 THERE IS EVEN ENOUGH MONEY IN THE BUCKET TO FUND ONE  
13 GRANT, THEN AT LEAST THIS GIVES THE INVESTIGATORS  
14 THAT INFORMATION. THEY CAN CONTINUE TO PROCESS AND  
15 PREPARE THEIR APPLICATIONS WITH THE UNDERSTANDING  
16 THAT THE PROBABILITY OF FUNDING IS GOING TO BE VERY  
17 LOW, BUT NEVERTHELESS THEY CAN MAKE A DECISION BASED  
18 ON THE PROBABILITY OF FUNDING WHICH WE ALL DO  
19 ANYWAY. THAT'S JUST PART OF THE GAME.

20 AGAIN, THIS IS TO AVOID THE SUDDEN AND  
21 WHAT SEEMS TO BE A VERY ABRUPT CLOSING OF THE VALVE.  
22 I'LL JUST THROW THAT OUT THERE FOR DISCUSSION.  
23 THANK YOU.

24 MR. SHEEHY: OS, THIS IS JEFF. IT IS  
25 ABRUPT, BUT THAT'S KIND OF WHERE WE ARE, RIGHT? SO

**BETH C. DRAIN, CA CSR NO. 7152**

1 I DON'T THINK -- I JUST DON'T KNOW HOW MUCH WE CAN  
2 PROMISE IN TERMS OF ADDITIONAL FUNDING. IF YOU LOOK  
3 AT THE NUMBERS, WE HAD 33 MILLION AND WE HAVE  
4 APPLICATIONS WORTH 76 MILLION, AND THEN WE HAVE  
5 APPLICATIONS ON TOP OF THAT COMING IN AS WELL. SO  
6 POTENTIALLY LOOKING AT THREE TIMES THE AMOUNT WE  
7 HAVE ALLOCATED -- APPLICATIONS WORTH THREE TIMES THE  
8 AMOUNT ALLOCATED. IT JUST DOESN'T SEEM LIKE WE CAN  
9 CREDIBLY SAY THAT THERE WILL BE ANY SIGNIFICANT  
10 PROGRAMMING PAST WHAT WE ARE DOING NOW.

11 AND THE OTHER QUESTION I MIGHT RAISE, AS  
12 THE TEAM HAS IT, IS WHAT IS THE ANTICIPATED, IF THEY  
13 KNOW AT THIS POINT, WHAT ARE THEY LOOKING AT AS  
14 POTENTIAL RETURN COMING BACK ON FUNDS THAT HAVE  
15 BEEN -- YEAH. WHAT ARE WE LOOKING AT IN TERMS OF  
16 FUNDS THAT ARE RETURNED FOR PROJECTS THAT HAVE  
17 FAILED TO MEET THEIR MILESTONES? YOU HAVE A NUMBER  
18 OR BALLPARK?

19 DR. STEWARD: BEFORE THEY ANSWER -- BEFORE  
20 CIRM TEAM ANSWERS THAT, CAN I JUST MAYBE ADDRESS  
21 WHAT YOUR FIRST POINT WAS, WHICH IS, AGAIN, I AGREE  
22 WITH EVERYTHING YOU SAID. AND THE ONLY THING THAT  
23 I'M DIFFERING ON IS A COMPLETE HALT VERSUS A PAUSE.  
24 WHAT I WOULD PROPOSE IS THAT WE SIMPLY INDICATE  
25 THAT, YES, THERE WON'T BE AN OPEN ROUND IN JULY OR

**BETH C. DRAIN, CA CSR NO. 7152**

1 AUGUST, BUT THAT THE SITUATION WOULD BE REEVALUATED  
2 IN SEPTEMBER AND, DEPENDING ON THE AVAILABILITY OF  
3 FUNDS, BY THAT TIME WE WILL KNOW THE OUTCOME OF THE  
4 ONES THAT ARE IN THE SYSTEM, THEN WE CAN DETERMINE  
5 WHETHER OR NOT TO HAVE ANOTHER ROUND OF FUNDING.

6 IT'S MAYBE A SMALL DIFFERENCE, BUT I THINK  
7 IT'S AN IMPORTANT DIFFERENCE IN TERMS OF PERCEPTION  
8 AS FAR AS GIVING SOME OPPORTUNITY AND THOUGHT TO HOW  
9 WE END THIS PROGRAM IN AN ORGANIZED WAY, LET'S CALL  
10 IT. THANK YOU.

11 CHAIRMAN THOMAS: WE HAVE COMMENTS IN  
12 ORDER FROM GABE AND THEN FROM GIL.

13 MR. THOMPSON: YES. THIS IS GABE  
14 THOMPSON, VICE PRESIDENT OF GRANTS AND OPERATIONS.  
15 WE HAVE AS OF TODAY APPROXIMATELY \$17.9 MILLION IN  
16 FUNDING THAT WE'VE ACCRUED THAT IS IN OUR  
17 UNALLOCATED BUCKET. AND SO THAT'S WHAT WE HAVE AS  
18 OF TODAY.

19 DR. SAMBRANO: SO THANK YOU. THIS IS GIL.  
20 SO JUST TO ADDRESS, OS, YOUR CONCERN, OUR INTENT  
21 WITH THIS MEMO WAS TO GIVE YOU AND OTHER BOARD  
22 MEMBERS A PICTURE OF WHERE WE BELIEVE WE ARE TODAY.  
23 BUT WITH THAT SAID, KNOWING THAT THINGS CAN CHANGE  
24 DEPENDING ON OUTCOMES AT NEXT WEEK'S GWG REVIEW IF  
25 NOTHING GETS RECOMMENDED, IF EVERYTHING GETS A

**BETH C. DRAIN, CA CSR NO. 7152**

1 THREE, THEN AND NO APPLICATIONS COME IN IN JUNE,  
2 THEN THE DECISION MAY BE TO KEEP THE JULY  
3 APPLICATION DEADLINE OPEN AND EXTEND IT UNTIL IT  
4 SEEMS APPROPRIATE.

5 BUT WE ALSO AGREE THAT IF THERE ARE  
6 CLEARLY FUNDS THAT BECOME AVAILABLE, EITHER THROUGH  
7 THE RETURNS LATER ON, THAT WE WOULD REOPEN THE  
8 APPLICATION WINDOW AND THAT OPPORTUNITY. BUT PART  
9 OF THIS WAS JUST TO KEEP YOU AS WELL AS THE PUBLIC  
10 INFORMED OF WHERE WE THINK WE ARE KNOWING THAT IT  
11 COULD CHANGE.

12 CHAIRMAN THOMAS: ARE THERE OTHER COMMENTS  
13 FROM MEMBERS OF THE BOARD? OKAY. THEN I THINK WE'D  
14 TAKE NOTE OF ALL THE COMMENTS WE'VE HAD. GIL, THANK  
15 YOU VERY MUCH FOR THE MEMO. WE WILL INDEED SEE WHAT  
16 HAPPENS IN THE SHORT TERM WITH THE GWG REVIEWS AND  
17 THE SUBMISSIONS, AND IT WILL SUPPLEMENT OUR THINKING  
18 ON THIS POINT ACCORDINGLY.

19 OKAY. THAT BRINGS US NOW TO PUBLIC  
20 COMMENT ON ANY TOPICS IN GENERAL. DO WE HAVE ANY  
21 MEMBERS OF THE PUBLIC EITHER HERE OR ON THE PHONE  
22 WHO WOULD LIKE TO SPEAK AT THIS POINT? HEARING  
23 NONE --

24 CONFERENCE OPERATOR: IF YOU'D LIKE TO ASK  
25 A QUESTION FROM THE PHONE, PRESS STAR ONE.

**BETH C. DRAIN, CA CSR NO. 7152**

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CHAIRMAN THOMAS: OKAY. HEARING NO PUBLIC COMMENT, THAT CONCLUDES THE BUSINESS OF TODAY'S APPLICATION REVIEW SUBCOMMITTEE. THANK YOU, EVERYBODY, FOR ATTENDING AND WE LOOK FORWARD TO THE NEXT MEETING, WHICH WILL BE TELEPHONIC IN JULY.

(THE PROCEEDING WAS THEN CONCLUDED AT 10:10 A.M.)

**REPORTER'S CERTIFICATE**

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE TELEPHONIC PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON JUNE 20, 2019, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CA CSR 7152  
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